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EMA/COMP/7038/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Amatuximab for the treatment of malignant mesothelioma

On 16 January 2014, orphan designation (EU/3/13/1222) was granted by the European Commission to Eisai Europe Limited, United Kingdom, for amatuximab for the treatment of malignant mesothelioma.

What is malignant mesothelioma?

Malignant mesothelioma is a cancer that affects the mesothelial cells (found on the inner linings of the organs), mainly in the pleura (the lining of the lungs) and in the peritoneum (the lining of the abdominal cavity). It is usually caused by exposure to asbestos. Mesothelioma of the pleura causes difficulty breathing and chest pain, and mesothelioma of the peritoneum causes ascites (a build-up of fluid in the abdomen) and abdominal pain.

Malignant mesothelioma is life-threatening because it may lead to bowel obstruction, heart or breathing problems and lung infections. Patients have very poor survival, only living for a year, on average, after diagnosis.

What is the estimated number of patients affected by the condition?

At the time of designation, malignant mesothelioma affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, the main treatment for malignant mesothelioma was surgery followed by chemotherapy (medicines to treat cancer) or radiotherapy (treatment with radiation). If the disease was too advanced for surgery, chemotherapy alone was used. Only one medicine, pemetrexed, was specifically authorised in the EU for the treatment of malignant pleural mesothelioma.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 512,200,000 (Eurostat 2013).

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The sponsor has provided sufficient information to show that amatuximab might be of significant benefit for patients with malignant mesothelioma because early clinical studies showed that it might improve the outcome for patients with this condition when used with standard-of-care treatment for malignant mesothelioma. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Amatuximab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure (called an antigen) that is found on certain cells in the body. Amatuximab has been designed to attach to mesothelin, a protein that is found in high amounts on the surface of mesothelioma cells. By attaching to mesothelin, amatuximab is expected to activate certain cells in the immune system (the body's natural defences), so that they kill the cancerous cells. This is expected to slow down the development of malignant mesothelioma.

What is the stage of development of this medicine?

The effects of amatuximab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with amatuximab in patients with malignant mesothelioma were ongoing.

At the time of submission, amatuximab was not authorised anywhere in the EU for malignant mesothelioma. Orphan designation of amatuximab had been granted in the United States for mesothelioma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 December 2013 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Amatuximab	Treatment of malignant mesothelioma
Bulgarian	аматуксимаб	Лечение на малигнен мезотелиом
Czech	Amatuximab	Léčba maligního mezoteliomu
Croatian	Amatuksimab	Liječenje malignog mezotelioma
Danish	Amatuximab	Behandling af malignt mesotheliom
Dutch	Amatuximab	Behandeling van maligne mesothelioom
Estonian	Amatuksimab	Pahaloomulise mesotelioomi ravi
Finnish	Amatuksimabi	Malignin mesotelioman hoito
French	Amatuximab	Traitement du mésothéliome malin
German	Amatuximab	Behandlung des malignen Mesothelioms
Greek	Αματουξιμάμπη	Θεραπεία κακοήθους μεσοθηλιώματος
Hungarian	Amatuximab	Rosszindulatú mesothelioma kezelése
Italian	Amatuximab	Trattamento del mesotelioma maligno
Latvian	Amatuksimabs	Ļaundabīgas mezoteliomas ārstēšana
Lithuanian	Amatuksimabas	Piktybinės mezoteliomos gydymas
Maltese	Amatuximab	Kura tal-mesoteljoma malinna
Polish	Amatuxymab	Leczenie złośliwego międzybłoniaka
Portuguese	Amatuximab	Tratamento do Mesotelioma maligno
Romanian	Amatuximab	Tratamentul mezoteliomului malign
Slovak	Amatuximab	Liečba malígneho mezoteliómu
Slovenian	Amatuksimab	Zdravljenje malignega mezotelioma
Spanish	Amatuximab	Tratamiento del mesotelioma maligno
Swedish	Amatuximab	Behandling av malignt mesoteliom
Norwegian	Amatuksimab	Behandling av malignt mesoteliom
Icelandic	Amatúximab	Meðferð við illkynja miðþekjuæxli

[1] At the time of designation